

IMMUNOTHERAPEUTICS FOR PEDIATRIC SOLID TUMORS

SUMMARY

The National Cancer Institute's Pediatric Oncology Branch seeks partners interested in licensing or collaborative research to co-develop new immunotherapeutic agents based on chimeric antigen receptor (CARs) for the treatment of pediatric solid tumors.

REFERENCE NUMBER

E-007-2014

PRODUCT TYPE

- Therapeutics

KEYWORDS

- adoptive cell therapy
- ALK
- anaplastic lymphoma kinase
- CD246
- chimeric antigen receptor
- CAR
- immunotherapy
- pediatric
- solid tumors

COLLABORATION OPPORTUNITY

This invention is available for licensing and co-development.

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DESCRIPTION OF TECHNOLOGY

Chimeric antigen receptors (CARs) are hybrid proteins consisting of an antibody binding fragment fused to protein signaling domains that cause T-cells which express the CAR to become cytotoxic. Once activated, these cytotoxic T-cells can selectively eliminate the cells which they recognize via the antibody binding fragment of the CAR. By engineering a T-cell to express a CAR that is specific for a certain cell surface protein, it is possible to selectively target those cells for destruction. This is a promising new

therapeutic approach known as adoptive cell therapy.

Anaplastic lymphoma kinase (ALK, CD246) is a tumor-associated antigen that is expressed on the cell surface of pediatric neuroblastomas and some non-small cell lung carcinomas (NSCLC). This technology from NCI's [Pediatric Oncology Branch](#) concerns the development of four (4) CARs, each comprising a different antibody binding fragment to ALK. The CARs, known individually as ALKCAR15, ALKCAR48, ALKCAR53 and ALKCAR58, can be used in adoptive cell therapy treatment for neuroblastoma and other solid tumors which overexpress ALK or variants thereof.

POTENTIAL COMMERCIAL APPLICATIONS

- Treatment of cancers associated with expression of ALK or variants thereof; Specific cancers include neuroblastoma, NSCLC and other solid tumors

COMPETITIVE ADVANTAGES

- High affinity of the ALKCAR15, ALKCAR48, ALKCAR53 and ALKCAR58 increases the likelihood of successful targeting;
- Targeted therapy decreases non-specific killing of healthy, essential cells, resulting in fewer non-specific side-effects and healthier patients

INVENTOR(S)

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DEVELOPMENT STAGE

- Pre-clinical (in vivo)

PUBLICATIONS

Orentas RJ, Lopomo P, Babbitt W, Vigny M, Mackall CL. 2013. "ALK (anaplastic lymphoma kinase, CD246) specific CARs: new immunotherapeutic agents for the treatment of pediatric solid tumors." *Journal for ImmunoTherapy of Cancer*, 1 (Suppl 1): P27

PATENT STATUS

- **U.S. Filed:** US Provisional Patent Application No. 61/865,845 filed 06 November 2013

RELATED TECHNOLOGIES

- E-104-2013
- E-291-2012

THERAPEUTIC AREA

- Cancer/Neoplasm